

Generating deeper and more durable BCMA CAR T cell responses in Multiple Myeloma through non-viral knockin/knockout multiplexed genome engineering

Grant Award Details

Generating deeper and more durable BCMA CAR T cell responses in Multiple Myeloma through non-viral knockin/knockout multiplexed genome engineering

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-13051

Investigator:

Name:	Julia Carnevale
Institution:	University of California, San Francisco
Type:	PI

Human Stem Cell Use: Somatic Cell

Award Value: \$1,463,368

Status: Pre-Active

Grant Application Details

Application Title: Generating deeper and more durable BCMA CAR T cell responses in Multiple Myeloma through non-viral knockin/knockout multiplexed genome engineering

Public Abstract: **Research Objective**

We will use integrated gene editing techniques to develop a new CAR-T cell therapy for multiple myeloma treatment

Impact

Develop an improved CAR-T cell therapy for patients with refractory multiple myeloma and a new manufacturing strategy that circumvents the costs and inefficiencies of viral production.

Major Proposed Activities

- Establish and optimize a CRISPR Cas9 editing strategy to generate combined non-viral TRAC-targeted BCMA CAR-T cells with RASA2 ablation.
- Evaluate key functional characteristics of TRAC-targeted BMCA CAR T cells with RASA2 ablation in vitro.
- In vivo evaluation of RASA2 KO TRAC-targeted BCMA CAR-T cells in immunocompromised mice xenografted with multiple myeloma.

Statement of Benefit to California: Multiple Myeloma (MM) is the second most common blood cancer, and currently there is no cure. The MM team at UCSF, home to the Grand Multiple Myeloma Translational Initiative, provides cutting edge care and offers hope through clinical trials to many Californians with refractory MM. We aim to develop an improved CAR-T cell therapy that will demonstrate deeper and more enduring MM responses in an early phase clinical trial here at UCSF, and ultimately will become accessible to all patients.

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